

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
10 September 2004 (10.09.2004)

PCT

(10) International Publication Number
WO 2004/076626 A2

(51) International Patent Classification⁷:

C12N

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(21) International Application Number:

PCT/US2004/004891

(22) International Filing Date: 19 February 2004 (19.02.2004)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

10/373,249 24 February 2003 (24.02.2003) US

(71) Applicant (for all designated States except US): GEN-
VEC, INC. [US/US]; 65 West Watkins Mill Road,
Gaithersburg, MD 20878 (US).

(72) Inventor; and

(75) Inventor/Applicant (for US only): BROUGH, Douglas;
E. [US/US]; 9121 Tulip Grove Road, Gaithersburg, MD
20879 (US).

(74) Agents: KISLING, Heather, R. et al.; Leydig, Voit &
Mayer, Ltd., Two Prudential Plaza, Suite 4900, 180 North
Stetson Av, Chicago, IL 60601-6780 (US).

(84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

— without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

WO 2004/076626 A2

(54) Title: MATERIALS AND METHODS FOR TREATING DISORDERS OF THE EAR

(57) Abstract: The invention is directed to a method of changing the sensory perception of an animal. The method comprises administering an expression vector comprising a nucleic acid sequence encoding an atonal-associated factor, which is expressed to produce the atonal-associated factor resulting in generation of hair cells that allow perception of stimuli in the inner ear. Also provided is a method of generating a hair cell in differentiated sensory epithelium *in vivo*. The method comprises contacting differentiated sensory epithelial cells with an adenoviral vector (a) deficient in one or more replication-essential gene functions of the E1 region and E4 region, (b) comprising a spacer in the E4 region, and (c) comprising a nucleic acid sequence encoding an atonal-associated factor. The nucleic acid sequence is expressed to produce the atonal-associated factor such that a hair cell is generated. An adenoviral vector encoding an atonal-associated factor also is provided.